

CUPA

The NYU Grossman School of Medicine Working Group on
Compassionate Use and Preapproval Access
A Project of the NYU Grossman School of Medicine Division of Medical Ethics

2021 Annual Report

2021 was a year full of debate and discussion around investigational medical products and wrestling with ethical questions such as who should get access to them outside of clinical trials. CUPA was ready to help with these questions, as we pursued our three-part mission of research, policy, and education. The group had a year defined by innovative problem-solving efforts, large-scale collaboration with regulatory and governmental bodies, and critical policy advising. We centered our work on addressing the inequities that define the investigational medicine landscape as we worked to ensure the fair and transparent treatment of all patients and to demystify non-trial access to investigational medical products.

CUPA—the group and its individual members—experienced much expansion and growth last year, adding two subgroups and multiple members to our roster.

- CUPA gained several new members: Sukhun Kang is a PhD student in strategy and entrepreneurship at the London Business School. Connie Law is a Residency Preceptor and Clinical Pharmacy Specialist at the Children’s Hospital of Philadelphia and Adam Motsney is an Investigational Drug Service Pharmacy Supervisor, also at the Children’s Hospital of Philadelphia. We are excited to add their expertise to the group!
- Kay Holcombe was elected to chair the board of directors for NORD, the National Organization for Rare Disorders.
- Jinsy Andrews was elected as the co-chair of the Northeast ALS Consortium (NEALS) for a four-year term.
- The Preapproval Access & Gene Therapy subgroup has existed informally for a few years, but now is a bona fide subgroup of CUPA. Its mission is to advance research, policy, and education regarding the ethical issues surrounding access to investigational gene-based interventions. In April 2021, Lisa Kearns, Ken Moch, Art Caplan, Tom Watson, Andrew McFadyen, Pat Furlong, Alison Bateman-House, and former member Carolyn Chapman published “[Gene therapy companies have an ethical obligation to develop expanded access policies](#)” in *Molecular Therapy*. In December, Lisa Kearns presented the paper’s findings to the Expanded Access Programmes Global Congress 2021 Americas. Please visit the [group’s webpage](#) for more information and a list of members.
- The Bioethics of Individualized Therapeutics subgroup (B.I.T.), formed in March 2021, brings together ethicists, clinicians, patient advocates, industry members, and research regulatory officials to study the ethical issues surrounding individualized therapeutic approaches for severely debilitating or life-threatening rare genetic diseases. The group seeks to advance understanding of the opportunities, challenges, and nascent best practices relevant to this emerging paradigm. In 2021, B.I.T.’s focus was on familiarizing itself with the current landscape of individualized therapeutics through a review of literature and conversations with a diverse array of experts in

the field. Additionally, group chair Alison Bateman-House and Lisa Kearns published [“Individualized therapeutics development for rare diseases: The current ethical landscape and policy responses”](#) in *Nucleic Acids Therapeutics*. In other activities, Lisa Kearns, Rafael Escandon, and Andrew McFadyen spoke at the 2021 Hope for Hasti Virtual Scientific Conference on a panel entitled, “The path to an N-of-1 clinical trial”; Alison Bateman-House was a panelist at the Chan Zuckerberg Initiative’s “‘N-of-1’ Gene Therapies: Bridging the scientific and societal considerations” meeting; and Lisa Kearns presented “Individualized therapeutics for rare diseases: Ethical concerns and the path forward” at Orphan Drugs & Rare Diseases Global Congress 2021 Americas.

CUPA members continue to publish widely in academic and lay publications, advancing research and policy about ethical issues related to preapproval access to medical products.

Research

Here are some selected publications from 2021; current CUPA member names are bolded.

- **Hayley Belli, Lesha Shah, and Alison Bateman-House**, along with Danielle Leach and former CUPA member Carolyn Riley Chapman, published [“A survey of pediatric hematologists/oncologists’ perspectives on single patient Expanded Access and Right to Try”](#) in *Medicine Access @ Point of Care*. Findings included a need for more education and support for physicians regarding both pathways to non-trial access to investigational drugs, in light of physicians’ “widespread willingness” to pursue access for patients.
- **Holly Fernandez Lynch** and co-authors Emily Largent and Andrew Peterson argue that integrating patient voices into the drug approval process shouldn’t lead to lowered standards for safety and efficacy in their viewpoint article [“FDA drug approval and the ethics of desperation,”](#) which appeared in *JAMA Internal Medicine*.
- Jamie Webb, **Lesha Shah**, and **Alison Bateman-House** published [“Siblings and discordant eligibility for gene therapy research: Considering parental requests for non-trial ‘compassionate use’”](#) in *Clinical Ethics*.

Policy

- [“Helpful lessons and cautionary tales: How should Covid-19 drug development and access inform approaches to non-pandemic diseases?”](#) by **Holly Fernandez Lynch, Art Caplan, Pat Furlong, and Alison Bateman-House**, was published online in the *American Journal of Bioethics* on Oct. 19. The article discusses concerns about adapting Covid-type drug development for non-public-health emergency contexts. This discussion will be continued during CUPA’s forthcoming webinar, titled “Where’s Our Operation Warp Speed? The Ethics and Realities of Implementing Pandemic Approaches for Non-Pandemic Diseases.” In this webinar, Paul Aliu (Novartis) and Dr. Bettina Ryll (Melanoma Patient Network Europe) will join **Holly Fernandez Lynch** and **Art Caplan** to discuss how the policy and funding commitments that shaped the U.S. response to Covid-19 might (or might not) be applied to other diseases with unmet need. Our highly subscribed event was originally scheduled for November 2021 but was hamstrung by technical difficulties, so we are

rescheduling for early this year. You can register for this free event by emailing Sage Gustafson at sage.gustafson@nyulangone.org!

- [“Implementation of the 21st Century Cures Act Expanded Access policies requirements,”](#) by **Sukhun Kang, Jennifer Miller**, Sungyong Chang, and Joseph Ross, was published on Aug. 24 in *Clinical Pharmacology & Therapeutics*.
- **Alison Bateman-House** and **Sage Gustafson**, along with John Massarelli, authored a guidance document for the World Health Organization titled “Clinical use of unproven interventions outside of research during a public health emergency.”
- **Holly Fernandez Lynch** and **Christopher Robertson** published [“Challenges in confirming drug effectiveness after early approval”](#) in *Science*.
- [“IRB review of expanded access protocols that collect real world data,”](#) by **Hayley Belli, Alison Bateman-House, Sage Gustafson**, and Jan Jaeger, was published in the PRIM&R (Public Responsibility in Medicine and Research) *Ampersand* blog on October 14. The post is a follow-up to a webinar they presented for PRIM&R titled “IRB Review of Expanded Access Protocols that Collect Real World Data: Considerations and Guidance.”

In 2021, CUPA members and their work were quoted and cited in many, many journals and news outlets, including (selected):

- Medical Ethics Advisor
- Molecular Therapy
- Medicine Access @ Point of Care
- Business Insider
- Real-World Evidence and Market Access Symposium
- JAMA Network
- JAMA Internal Medicine
- Stat
- Stat Plus
- Journal of Community Health
- Pink Sheet/Informa Pharma Intelligence
- New York Times
- Hashtags Center Report
- Clinical Microbiology and Infection Online
- Nature Medicine
- American Journal of Bioethics
- Denver Law Review
- PRIM&R
- Acta Paediatrica
- Pediatrics
- Arizona Health Law & Policy Program
- BioCentury
- Health Affairs
- Medscape
- Journal of Law, Medicine & Ethics

Education

2021 was the second year of CUPA’s online course “Access to Investigational Medical Products: Clinical Trials, Expanded Access, and Right to Try,” taught by **Alison Bateman-House** and **Sage Gustafson** and offered through the University of Arizona Health Law Program. This past year, CUPA and the University of Arizona awarded full scholarships to 3 CUPA Patient Advocate Scholars: Michael Raymond, Lori Larson, and Elizabeth Volz. The course will run again this summer. To learn more, contact Dr. Tara Sklar at law-healthcare@email.arizona.edu.

CUPA launched a paid undergraduate internship program in 2021, designed to educate students from backgrounds underrepresented in bioethics about the ethical complexities of preapproval access and to prepare them for careers in academic research. CUPA is truly lucky with our first outstanding intern, Rei Marshall. She is a senior at the University of Washington in Seattle, pursuing a BS in public health–global

health, with a special interest in health equity and access. Year one of the internship was generously funded by an unrestricted gift from Bionical Emas. To make a gift to support this internship program, please contact Alison.Bateman-House@nyulangone.org.

Our members participated in many education-focused events this past year, sponsored by both CUPA and other entities, including (selected):

- American Academy of Neurology's Neurology Today
- American Society for Bioethics & Humanities conference
- American Society of Gene & Cell Therapy Policy Summit
- BioCentury
- BridgeBio
- Consortium of Universities for Global Health 2021
- DIA Bioethics Community
- DIA Global Annual Meeting
- Early and Managed Access Programmes Europe digital conference
- Emory University
- FDA
- Forum for Global Health Ethics
- Galien Foundation/Jerusalem Ethics Forum
- Herman & Walter Samuelson Children's Hospital at Sinai
- I Am ALS
- International Federation of Associations of Pharmaceutical Physicians and Pharmaceutical Medicine Academy
- International Neonatal Consortium
- Jackson Laboratory and Hope for Hasti's Virtual Scientific Conference on Cornelia de Lange Syndrome (CdLS)
- John B. Corser Memorial Symposium
- Johnson & Johnson
- Legal Liability for Allocation of Scarce Resources in Health Care in the Covid-19 Pandemic conference
- Medpage Today
- Medscape
- New York Academy of Science
- North Texas Chapter of the Association of Clinical Research Professionals (ACRP)
- Operationalize: Expanded Access Programs
- Orphan Disease Center's 10th Anniversary Symposium
- Orphan Drugs & Rare Diseases Global Congress Americas—East Coast
- Parent Project Muscular Dystrophy
- Pediatric Oncology Experimental Therapeutics Investigators' Consortium (POETIC) winter meeting
- PRIM&R, Public Responsibility in Medicine and Research
- Real-World Evidence and Market Access Symposium
- Society for Clinical Trials Annual Meeting
- Stat
- [University of Arizona Health Law & Policy Program](#)
- University of Toronto Joint Centre for Bioethics
- World Orphan Drug Congress USA
- 40th Annual Philippa Harris Lecture on Bioethical Issues in Cancer
- 24 Hours of Rare

We are so glad to have your continued support and interest in CUPA's mission! We believe that our work on the ethical issues surrounding preapproval access is of vital, practical import to patients, industry, clinicians, and the public. If you or someone you know would like to receive our free monthly newsletter, contact Sage Gustafson (sage.gustafson@nyulangone.org). If you would like to support CUPA—which operates solely on donations—please contact Lisa Kearns (lisa.kearns@nyulangone.org).

We're excited for what we have planned for 2022, so please stay tuned!

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